Aldeyra Therapeutics Announces Positive Top-Line Symptom and Sign Results from Run-In Cohort of Phase 3 TRANQUILITY Trial in Dry Eye Disease

January 7, 2021

- Statistical Significance of Reproxalap Over Vehicle Achieved for Ocular Redness, an FDA-Approvable Sign, and Clinical Symptoms of Ocular Dryness and Discomfort
- Acute Improvement in Ocular Redness and Symptom Scores Demonstrated Within Minutes of Reproxalap Administration in Dry Eye Chamber
- Main Cohort of TRANQUILITY Expected to Begin Enrollment in February 2021, Following Completion of Tear RASP Analysis and Finalization of Trial Design
- Management to Host Conference Call at 8:00 a.m. ET Today

LEXINGTON, Mass.--(BUSINESS WIRE)--Jan. 7, 2021-- Aldeyra Therapeutics, Inc. (Nasdaq: ALDX) (Aldeyra) today announced positive top-line symptom, redness, and Schirmer's test results from the run-in cohort of the Phase 3 TRANQUILITY clinical trial in patients with dry eye disease.

The double-masked, single-center, parallel-group run-in cohort enrolled 23 patients: 12 patients were randomized to receive 0.25% reproxalap ophthalmic solution and 11 patients were randomized to receive vehicle ophthalmic solution. Patients received four doses one day prior to and two doses on the day of exposure to a 90-minute dry eye chamber with minimal humidity, high airflow, and forced visual tasking.

Over all time points in aggregate in the dry eye chamber, reproxalap was observed to be statistically superior to vehicle for the two assessed symptoms, visual analog scale (VAS) ocular dryness score ($p = 0.001$) and ocular discomfort score ($p < 0.0001$) in the run-in cohort of TRANQUILITY. Consistent with previously announced allergen chamber Phase 2 clinical trial results, reproxalap demonstrated statistically significant improvement over vehicle ($p = 0.03$) in ocular redness, an objective sign of dry eye disease. Improvement in ocular symptoms and redness occurred within minutes after reproxalap dosing. Following acute dosing on the day prior to the dry eye chamber, Schirmer test scores were directionally in favor of reproxalap over vehicle, and reproxalap was statistically superior to vehicle in improvement in VAS dryness score ($p = 0.003$), ocular discomfort 4-symptom questionnaire (OD4SQ) dryness score ($p = 0.006$), OD4SQ grittiness score ($p = 0.006$), and OD4SQ discomfort score ($p = 0.003$). Consistent with clinical experience in over 1,100 patients, no adverse findings on safety assessments were observed, and reproxalap was well-tolerated.

"The symptom improvement observed in the run-in cohort of TRANQUILITY announced today support the first-line potential use of reproxalap in dry eye disease, and represent the first results from an ophthalmic solution for chronic use that demonstrate activity acutely following drug administration," stated Todd C. Brady, M.D., Ph.D., President and Chief Executive Officer of Aldeyra. "The activity of reproxalap in reducing ocular redness, initially demonstrated in the allergen chamber Phase 2 clinical trial, was also observed in the dry eye chamber run-in results of TRANQUILITY, and we look forward to initiating enrollment of the main cohort."

Among many patients and physicians, current dry eye disease therapies are considered inadequate. Discontinuation rates for lifitegrast and cyclosporine, which currently comprise standard of care, exceed 60% within 12 months of initiation of therapy, in part due to delayed onset of effect.[1]

"The potential for patients to experience acute symptomatic relief and observe rapid improvement in ocular redness after the initiation of therapy represents a new possible paradigm in dry eye disease treatment and could offer significant clinical advantage over existing therapies, which often require weeks of drug administration before patients experience even moderate improvement," stated Victor Perez, M.D., Professor of Ophthalmology at Duke University School of Medicine and a member of Aldeyra's Anterior Segment Scientific Advisory Board.

The main cohort of TRANQUILITY is expected to begin enrollment in February 2021, following completion of tear RASP analysis from the run-in cohort and confirmation of endpoints and number of subjects. Results from TRANQUILITY are expected in the second half of 2021. A second Phase 3 clinical trial, TRANQUILITY-2, is expected to initiate in the first quarter of 2021.

Conference Call Information

Aldeyra will host a conference call to discuss this announcement today, Thursday, January 7, 2021, at 8:00 a.m. ET. The dial-in numbers are (866) 211-4098 for domestic callers and (647) 689-6613 for international callers. The Conference ID is 7076648. A live webcast of the conference call will also be available on the Investor Relations section of the Aldeyra Therapeutics website at https://ir.aldeyra.com. Presentation slides will be available on the investor relations page approximately 30 minutes prior to the start of the conference call and webcast.

After the live webcast, the event will remain archived on the Aldeyra Therapeutics website for 90 days.

About Reproxalap

Reproxalap is a novel small-molecule immune-modulating covalent inhibitor of RASP (reactive aldehyde species), which are elevated in ocular and systemic inflammatory disease. Reproxalap's mechanism of action has been validated with the demonstration of statistically significant and clinically relevant activity in multiple physiologically distinct late-phase clinical indications. Reproxalap is currently in Phase 3 clinical development as a 0.25% ophthalmic solution for the treatment of dry eye disease and allergic conjunctivitis, two ocular inflammatory diseases that often occur together in the same patient.

About Dry Eye Disease

Dry eye disease is a common inflammatory disease estimated to affect 34 million or more adults in the United States.[2] The disease is characterized by insufficient moisture and lubrication in the anterior surface of the eye, leading to dryness, inflammation, pain, discomfort, irritation, diminished quality of life, and in severe cases, permanent vision impairment. Among many physicians and patients, existing therapy for dry eye disease is generally regarded as inadequate and often requires weeks or months to demonstrate activity. In patients with dry eye disease, pro-inflammatory RASP may contribute to ocular inflammation and changes in tear lipid composition. By diminishing RASP levels, Aldeyra's lead RASP inhibitor reproxalap represents a novel and differentiated approach for the treatment of the symptoms and signs of dry eye disease.

About Aldeyra Therapeutics

Aldeyra Therapeutics is a clinical-stage biotechnology company focused on the development of novel therapies with the potential to improve the lives of patients with immune-mediated diseases. Two of the company's lead investigational compounds, reproxalap and ADX-629, target RASP (reactive aldehyde species), which are elevated in ocular and systemic inflammatory disease and result in cytokine release via activation of a broad array of inflammatory factors, including NF-κB, inflammasomes, and Scavenger Receptor A. Reproxalap is being evaluated in Phase 3 clinical trials in patients with dry eye disease and allergic conjunctivitis. The company's clinical pipeline also includes ADX-2191, a dihydrofolate reductase inhibitor in Phase 3 testing for proliferative vitreoretinopathy, and ADX-1612, a chaperome inhibitor in development for COVID-19 and ovarian cancer. For more information, visit https://www.aldeyra.com/ and follow us on LinkedIn, Facebook, and Twitter.

Safe Harbor Statement

This release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the initial clinical results from the run-in cohort of the Phase 3 TRANQUILITY Trial and expectations regarding the main cohort of TRANQUILITY and the TRANQUILITY-2 Trial. Aldeyra intends such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995. In some cases, you can identify forward-looking statements by terms such as, but not limited to, "may," "might," "will," "objective," "intend," "should," "could," "can," "would," "expect," "believe," "anticipate," "project," "on track," "scheduled," "target," "design," "estimate," "predict," "potential," "aim," "plan" or the negative of these terms, and similar expressions intended to identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions, and uncertainties. Aldeyra is at an early stage of development and may not ever have any products that generate significant revenue. All of Aldeyra's development timelines may be subject to adjustment depending on recruitment rate, regulatory review, preclinical and clinical results, and other factors that could delay the initiation or completion of clinical trials. Important factors that could cause actual results to differ materially from those reflected in Aldeyra's forward-looking statements include, among others, the timing of enrollment, commencement and completion of Aldeyra's clinical trials, the timing and success of preclinical studies and clinical trials conducted by Aldeyra and its development partners; updated or refined data based on Aldeyra's continuing review and quality control analysis of clinical data, Aldeyra's ability to design clinical trials with protocols and endpoints acceptable to applicable regulatory authorities; delay in or failure to obtain regulatory approval of Aldeyra's product candidates; the ability to maintain regulatory approval of Aldeyra's product candidates, and the labeling for any approved products; the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical or clinical trials, will not be replicated or will not continue in ongoing or future studies or clinical trials involving Aldeyra's product candidates; the risk that the results from smaller clinical trials or portions of clinical trials may not accurately predict results of larger scale trials or the remainder of a clinical trial; the scope, progress, expansion, and costs of developing and commercializing Aldeyra's product candidates; uncertainty as to Aldeyra's ability to commercialize (alone or with others) Aldeyra's product candidates following regulatory approval, if any; the size and growth of the potential markets and pricing for Aldeyra's product candidates and the ability to serve those markets; Aldeyra's expectations regarding Aldeyra's expenses and revenue, the sufficiency or use of Aldeyra's cash resources and needs for additional financing; political, economic, legal, social and health risks, including the recent COVID-19 outbreak and subsequent public health measures, that may affect Aldeyra's business or the global economy; the rate and degree of market acceptance of any of Aldeyra's product candidates; Aldeyra's expectations regarding competition; Aldeyra's anticipated growth strategies; Aldeyra's ability to attract or retain key personnel; Aldeyra's limited sales and marketing infrastructure; Aldeyra's ability to establish and maintain development partnerships; Aldeyra's ability to successfully integrate acquisitions into its business; Aldeyra's expectations regarding federal, state and foreign regulatory requirements; regulatory developments in the United States and foreign countries; Aldeyra's ability to obtain and maintain intellectual property protection for its product candidates; the anticipated trends and challenges in Aldeyra's business and the market in which it operates; and other factors that are described in the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of Aldeyra's Annual Report on Form 10-K for the year ended December 31, 2019 and Aldeyra's Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, which are on file with the Securities and Exchange Commission (SEC) and available on the SEC's website at https://www.sec.gov/. Additional factors may be described in those sections of Aldeyra's Annual Report on Form 10-K for the year ended December 31, 2020, expected to be filed with the SEC in the first quarter of 2021.

In addition to the risks described above and in Aldeyra's other filings with the SEC, other unknown or unpredictable factors also could affect Aldeyra's results. No forward-looking statements can be guaranteed and actual results may differ materially from such statements. The information in this release is provided only as of the date of this release, and Aldeyra undertakes no obligation to update any forward-looking statements contained in this release on account of new information, future events, or otherwise, except as required by law.

[1] White DE, Zhao Y, Ogundele A, et al. Real-World Treatment Patterns of Cyclosporine Ophthalmic Emulsion and Lifitegrast Ophthalmic Solution Among Patients with Dry Eye. Clin Ophthalmol. 2019;13:2285-2292.

[2] Paulsen AJ, Cruickshanks KJ, Fischer ME, et al. Dry eye in the beaver dam offspring study: prevalence, risk factors, and health-related quality of life. Am J Ophthalmol. 2014;157(4):799-806. doi:10.1016/j.ajo.2013.12.023.

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